UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of Earliest Event Reported): **November 10, 2017**

Blueprint Medicines Corporation

(Exact name of registrant as specified in its charter)

Delaware	**001-37359**	**26-3632015**
(State or other jurisdiction of incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

38 Sidney Street, Suite 200 **Cambridge, Massachusetts**	**02139**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: **(617) 374-7580**

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☑

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☑

Item 7.01 Regulation FD Disclosure.

On November 10, 2017, Blueprint Medicines Corporation (the "Company") issued a press release announcing new data from its ongoing Phase 1 clinical trial evaluating BLU-285 for the treatment of advanced gastrointestinal stromal tumors. The data were presented on Friday, November 10, 2017 in an oral presentation at the 22nd Connective Tissue Oncology Society ("CTOS") Annual Meeting in Maui, Hawaii. A copy of the press release is furnished as Exhibit 99.1 to this Current Report on Form 8-K, and a copy of the presentation at the CTOS Annual Meeting is furnished as Exhibit 99.2 to this Current Report on Form 8-K.

The information in Item 7.01 of this Current Report on Form 8-K, including Exhibits 99.1 and 99.2, is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
99.1	Press release issued by Blueprint Medicines Corporation on November 10, 2017
99.2	Presentation by Blueprint Medicines Corporation at the CTOS Annual Meeting on November 10, 2017

EXHIBIT INDEX

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

BLUEPRINT MEDICINES CORPORATION

Date: November 13, 2017

By: /s/ Tracey L. McCain
Tracey L. McCain
Chief Legal Officer

Blueprint Medicines Announces New Data from Ongoing Phase 1 Clinical Trial of BLU-285 in Patients with Advanced Gastrointestinal Stromal Tumors Showing Strengthened Clinical Activity across Spectrum of KIT and PDGFRα Genotypes

– Global, Randomized Phase 3 Trial in Third-Line GIST on Track to Initiate in First Half 2018 –
– New Cohort in Second-Line GIST Added to Ongoing Phase 1 Trial –

CAMBRIDGE, Mass., November 10, 2017 – Blueprint Medicines Corporation (NASDAQ: BPMC), a leader in discovering and developing targeted kinase medicines for patients with genomically defined diseases, today announced new Phase 1 clinical data for BLU-285, a potent and highly selective KIT and PDGFRα inhibitor in development as a potential treatment for patients with advanced gastrointestinal stromal tumors (GIST). The data confirm and build upon data previously presented for BLU-285 in patients with advanced GIST, demonstrating robust clinical activity and a favorable safety profile. The data showed 67 percent of patients with heavily pretreated KIT-driven GIST treated with 300 to 400 mg of BLU-285 once daily (QD) had radiographic tumor reductions. In this population, the data also showed an objective response rate (ORR) of 17 percent and median progression free survival (PFS) of 11.5 months. In patients with PDGFRα-D842 driven GIST, the data showed an ORR of 71 percent and an estimated 12-month PFS of 78 percent. BLU-285 was well-tolerated, and most adverse events (AEs) reported by investigators were Grade 1 or 2. The data will be presented today in an oral presentation at the Connective Tissue Oncology Society 2017 Annual Meeting in Maui, Hawaii.

As previously announced, Blueprint Medicines plans to pursue expedited development of BLU-285 in patients with PDGFRα D842V-driven GIST, and the Company is on track to initiate a global, randomized Phase 3 clinical trial of BLU-285 in third-line GIST in the first half of 2018, with the goal of supporting registration of BLU-285 in a broader GIST patient population. In addition, based on the strength of the data in patients with KIT-driven GIST, Blueprint Medicines announced today an expansion of the ongoing Phase 1 trial. The Company recently increased the enrollment target for patients previously treated with imatinib and at least one additional tyrosine kinase inhibitor (TKI) from 50 to 100 patients and added a new cohort to evaluate BLU-285 in second-line GIST.

“The updated BLU-285 data show robust clinical activity in a broad population of patients with advanced GIST,” said Michael Heinrich, M.D., Professor of Medicine at Oregon Health & Science University and an investigator on the clinical trial. “In patients with PDGFRα-driven advanced GIST, the data for BLU-285 continue to be remarkable, particularly considering these patients have no effective treatment options. Importantly, I’m also very encouraged to see radiographic tumor reductions in two-thirds of patients with heavily pretreated GIST across all common KIT genotypes, as well as prolonged progression free survival in this population. Overall, these clinical results confirm recently published preclinical data showing activity across a spectrum of disease-relevant mutations and support clinical development of BLU-285 in a broad patient population.”

“We are grateful to the patients, families and investigators who have contributed to the Phase 1 clinical trial to date, and we are committed to advancing the clinical development of BLU-285 quickly and responsibly,” said Andy Boral, M.D., Chief Medical Officer of Blueprint Medicines. “We continue to plan for expedited development of BLU-285 in patients with PDGFRα D842V-driven GIST, and we look forward to working with the FDA under the Breakthrough Therapy Designation program to determine the fastest path forward for BLU-285 in this population. In addition, the objective responses and prolonged progression free survival we are seeing in patients with heavily pretreated KIT-driven GIST give us increased confidence in our approach to expand development of BLU-285 in third- and second-line treatment. Overall, we believe our data-driven clinical development strategy will enable productive interactions with global regulatory authorities over the course of 2018.”

New Data from the Ongoing Phase 1 Clinical Trial of BLU-285 in Advanced GIST

As of the data cutoff date of October 11, 2017, 116 patients had been treated with BLU-285 in the dose escalation and expansion portions of the Phase 1 clinical trial at eight dose levels (ranging from 30 mg QD to 600 mg QD), including 76 patients with KIT-driven GIST, 39 patients with PDGFRα-driven GIST, and one patient with KIT/PDGFRα wild-type GIST. The median number of prior TKI regimens was four for patients with KIT-driven GIST (ranging from two to 11), and one for patients with PDGFRα-driven GIST (ranging from zero to six). Among patients with KIT-driven GIST, 64 patients (83 percent) previously received regorafenib.

Blueprint Medicines has selected 300 mg QD as the recommended part 2 dose (RP2D) for the expansion portion of the clinical trial, with an option for investigators to escalate patients to the maximum tolerated dose (MTD) of 400 mg QD following two treatment cycles.

Safety Data:

As of the data cutoff date, BLU-285 was generally well-tolerated. Most AEs reported by investigators were Grade 1 or 2. Across all grades, the most common treatment-emergent AEs reported by investigators (≥20 percent) included nausea (56 percent), fatigue (53 percent), periorbital edema (43 percent), vomiting (41 percent), edema peripheral (34 percent), anemia (31 percent), diarrhea (31 percent), increased lacrimation (30 percent), cognitive effects (30 percent), decreased appetite (28 percent), dizziness (23 percent), constipation (22 percent) and hair color changes (22 percent). Cognitive effects are an aggregated category of individual cognitive events, each of which was observed in fewer than 20 percent of patients. Investigators reported treatment-related Grade ≥3 AEs in 39 patients (34 percent), including anemia (9 percent), fatigue (7 percent), hypophosphatemia (4 percent), nausea (4 percent) and cognitive effects (3 percent). Six patients (5 percent) discontinued treatment with BLU-285 due to AEs. An additional 43 patients discontinued treatment, with 40 patients due to progressive disease and three patients who withdrew consent. Among all 116 enrolled patients, 67 remained on treatment as of the data cutoff date.

Clinical Activity Data:

As of the data cutoff date, 30 patients with KIT-driven GIST treated at 300 to 400 mg QD were evaluable for response assessments. In addition, 31 patients with PDGFRα D842-driven GIST at all doses were evaluable for response assessments, including 29 patients with a D842V mutation and two patients with other D842 mutations. Two patients with a PDGFRα exon 14 mutation were excluded from analyses of clinical activity. Patients were evaluable if they had at least one centrally reviewed radiographic scan, and all reported data are based on blinded central radiology review as per modified Response Evaluation Criteria in Solid Tumors version 1.1 (mRECIST 1.1 criteria) for GIST. Radiographic scans were also assessed by Choi criteria, a supportive method of response assessment in soft tissue sarcomas that has been shown to be predictive of improved prognosis in patients with advanced GIST.

Patients with heavily pretreated KIT-driven GIST treated at doses of 300 to 400 mg QD

- Centrally assessed radiographic tumor reductions were observed in 20 of 30 evaluable patients (67 percent) across all common KIT genotypes, including mutations in exons 9, 11, 13, 14 and 17, confirmed by archival tumor biopsy and circulating tumor DNA.
- By mRECIST 1.1 criteria, five patients had a partial response (PR) (three confirmed, two pending confirmation), and 18 patients had stable disease (SD), representing an ORR of 17 percent and a disease control rate (DCR) of 77 percent.
- By Choi criteria, 16 patients had a PR and seven patients had SD, representing an ORR of 53 percent and a DCR of 77 percent.
- Median PFS was 11.5 months.
- In contrast, historical data showed a zero percent ORR and median PFS of 1.8 months in patients with TKI-resistant advanced GIST re-treated with imatinib in a third-line or later setting.[1]

Patients with PDGFRα-driven GIST

- Centrally assessed radiographic tumor reductions were observed in all 31 evaluable patients.
- By mRECIST 1.1 criteria, one patient had a complete response (CR) (pending confirmation), 21 patients had a PR (18 confirmed, three pending confirmation), and nine patients had SD, representing an ORR of 71 percent and a DCR of 100 percent.
- By Choi criteria, one patient had a CR and 30 patients had a PR, representing an ORR of 100 percent.
- Median PFS was not reached, and 12-month PFS was estimated to be 78 percent.
- In contrast, historical data showed a zero percent ORR and median PFS of 2.8 months in patients with PDGFRα D842V-driven GIST treated with imatinib.[2]

About the Phase 1 Clinical Trial for BLU-285 in PDGFRα-Driven and KIT-Driven GIST

The Phase 1 clinical trial of BLU-285 is designed to evaluate the safety and tolerability of BLU-285 in adults with advanced GIST. The trial consists of two parts, a dose-escalation portion and an expansion portion. The dose-escalation portion is complete, and the MTD was determined to be 400 mg QD. Blueprint Medicines has selected 300 mg QD as the RP2D for the dose expansion portion of the trial. The expansion portion is actively enrolling patients in three defined cohorts at the RP2D consisting of (1) a cohort of patients with a PDGFRα D842V mutation, regardless of line of therapy, (2) a cohort of patients who have received imatinib and at least one other KIT-directed TKI and (3) a cohort of patients who have received imatinib and no other TKI. Trial objectives include assessing response, pharmacokinetics and pharmacodynamic measures. All response assessments use blinded, central radiology review. The three expansion cohorts of the trial are designed to enroll a total of approximately 200 patients at multiple sites in the United States, United Kingdom and European Union. Please refer to www.clinicaltrials.gov for additional details related to this Phase 1 clinical trial (NCT02508532). Patients and physicians may contact the study director for more information about this Phase 1 clinical trial at studydirector@blueprintmedicines.com.

About GIST

GIST is the most common sarcoma, or tumor of bone or connective tissue, of the gastrointestinal (GI) tract. Tumors arise from cells in the wall of the GI tract and occur most often in the stomach or small intestine. Most patients are diagnosed between the ages of 50-80, and diagnosis is typically triggered by GI bleeding, incidental findings during surgery or imaging and, in rare cases, tumor rupture or GI obstruction. Approximately 80 percent of GIST patients have KIT-driven GIST, and Blueprint Medicines estimates that KIT exon 17 mutations occur in approximately 90 percent of GIST patients with KIT-driven GIST following treatment with at least two TKIs. Approximately five percent of all advanced GIST cases are driven by D842V mutant PDGFRα. Patients diagnosed with GIST at an early stage may undergo surgery. For patients with KIT-driven GIST, treatment with the currently approved frontline therapy typically leads to treatment resistance and disease progression. Treatment options for KIT-driven GIST patients whose disease progresses or develops resistance are currently limited, with approved therapies providing a progression free survival of up to six months and a response rate between five percent and seven percent. There are no effective treatment options for patients with PDGFRα D842V-driven GIST, and progression often occurs in as little as three months with available treatment options.

About BLU-285

BLU-285 is an orally available, potent and highly selective inhibitor of KIT and PDGFRα. Preclinical data have shown that BLU-285 is active across a broad spectrum of KIT and PDGFRα mutations, including PDGFRα D842V and KIT exon 17 mutations for which there are limited or no effective treatment options. Blueprint Medicines is initially developing BLU-285, an investigational medicine, for the treatment of patients with advanced GIST and advanced systemic mastocytosis. BLU-285 was discovered by Blueprint Medicines' research team leveraging its proprietary compound library, and the Company retains worldwide development and commercialization rights for BLU-285.

In June 2017, BLU-285 received Breakthrough Therapy Designation from the FDA for the treatment of patients with unresectable or metastatic GIST harboring the PDGFRα D842V mutation. The FDA's Breakthrough Therapy Designation is intended to expedite the development and review of a drug candidate intended to treat a serious or life-threatening disease or condition, when preliminary clinical evidence indicates that the drug candidate may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. Previously, the FDA granted orphan drug designation to BLU-285 for the treatment of GIST.
The FDA also granted Fast Track designation to BLU-285 for the treatment of patients with unresectable or metastatic GIST that progressed following treatment with imatinib and a second TKI and for the treatment of patients with unresectable or metastatic GIST in patients with the PDGFRα D842V mutation regardless of prior therapy. In addition, the European Medicines Agency has granted orphan drug designation to BLU-285 for the treatment of GIST.

About Blueprint Medicines

Blueprint Medicines is developing a new generation of targeted and potent kinase medicines to improve the lives of patients with genomically defined diseases. Its approach is rooted in a deep understanding of the genetic blueprint of cancer and other diseases driven by the abnormal activation of kinases. Blueprint Medicines is advancing four programs in clinical development for subsets of patients with gastrointestinal stromal tumors, hepatocellular carcinoma, systemic mastocytosis, non-small cell lung cancer, medullary thyroid cancer and other advanced solid tumors, as well as multiple programs in research and preclinical development. For more information, please visit www.blueprintmedicines.com.

References

[1] Kang YK, Ryu MH, Ryoo BY, et al. Resumption of imatinib to control metastatic or unresectable gastrointestinal stromal tumours after failure of imatinib and sunitinib (RIGHT): a randomised, placebo-controlled, phase 3 trial. Lancet Oncol. 2013;14(12):1175–82.
2 Cassier PA, Fumagalli E, Rutkowski P, et al. Outcome of Patients with Platelet-Derived Growth Factor Receptor Alpha–Mutated Gastrointestinal Stromal Tumors in the Tyrosine Kinase Inhibitor Era. Clin Cancer Res. 2012;18(16):4458–64.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding plans and timelines for the clinical development of BLU-285, including plans and timelines for pursuing expedited development in patients with a PDGFRα D842V mutation and plans and timelines for the initiation of a global, randomized Phase 3 clinical trial of BLU-285 in third-line GIST; Blueprint Medicines' ability to implement its clinical development plans for BLU-285 in advanced GIST; expectations regarding current and future interactions with global regulatory authorities, including the FDA, and the impact of Breakthrough Therapy Designation on the development of BLU-285; and Blueprint Medicines' strategy, business plans and focus. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks and uncertainties related to the delay of any current or planned clinical trials or the development of Blueprint Medicines' drug candidates, including BLU-285, BLU-554 and BLU-667; Blueprint Medicines' advancement of multiple early-stage efforts; Blueprint Medicines' ability to successfully demonstrate the safety and efficacy of its drug candidates; the preclinical and clinical results for Blueprint Medicines' drug candidates, which may not support further development of such drug candidates; and actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials; Blueprint Medicines' ability to develop and commercialize companion diagnostic tests for its current and future drug candidates, including companion diagnostic tests for BLU-554 for FGFR4-driven HCC, BLU-285 for PDGFRα D842V-

driven GIST and BLU-667 for RET-driven non-small cell lung cancer; and the success of Blueprint Medicines' cancer immunotherapy collaboration with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc. These and other risks and uncertainties are described in greater detail in the section entitled “Risk Factors” in Blueprint Medicines’ Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, as filed with the Securities and Exchange Commission (SEC) on October 31, 2017, and other filings that Blueprint Medicines may make with the SEC in the future. Any forward-looking statements contained in this press release represent Blueprint Medicines’ views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Blueprint Medicines explicitly disclaims any obligation to update any forward-looking statements.

Media and Investor Relations Contacts

Kristin Hodous
617 714 6674
khodous@blueprintmedicines.com

Jim Baker
617 844 8236
jbaker@blueprintmedicines.com

Clinical activity of BLU-285, a highly potent and selective KIT/PDGFRα inhibitor designed to treat gastrointestinal stromal tumor (GIST)

Michael Heinrich[1], Robin Jones[2], Margaret von Mehren[3], Patrick Schöffski[4], Sebastian Bauer[5], Olivier Mir[6], Philippe A. Cassier[7], Ferry Eskens[8], Hongliang Shi[9], Terri Alvarez-Diez[9], Oleg Schmidt-Kittler[9], Mary Ellen Healy[9], Beni B. Wolf[9], Suzanne George[10]

[1]Knight Cancer Institute, OHSU, Portland, OR, USA; [2]Royal Marsden Hospital/Institute of Cancer Research, London, UK; [3]Fox Chase Cancer Center, Temple University Health System, Philadelphia, USA; [4]University Hospitals Leuven, Department of General Medical Oncology, Leuven Cancer Institute, Leuven, Belgium; [5]West German Cancer Center, University Hospital, Essen, Germany; [6]Gustave Roussy, Villejuif, France; [7]Centre Leon Berard, Lyon, France; [8]Erasmus MC Cancer Institute, Rotterdam, Netherlands; [9]Blueprint Medicines, Cambridge, MA, USA; [10]Dana-Farber Cancer Center, Boston, MA, USA

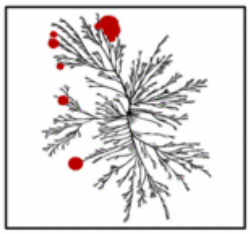
Abstract no: 2803523, CTOS 2017 Maui, Hawaii. Presented by Dr. Michael Heinrich

Disclosures

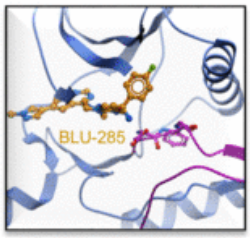
- BLU-285 is an investigational agent discovered and currently in development by Blueprint Medicines Corporation (Blueprint Medicines)
- Dr. Michael Heinrich is an investigator for Blueprint Medicines' ongoing Phase 1 study in unresectable gastrointestinal stromal tumor
- Dr. Michael Heinrich has the following disclosures:
  - Consultant: Blueprint Medicines, Novartis, MolecularMD, Deciphera
  - Equity interest: MolecularMD
  - Research funding: Blueprint Medicines, Deciphera, Ariad
  - Expert testimony: Novartis
  - Patents: four patents on diagnosis and treatment of PDGFRα-mutant GIST

BLU-285: highly selective targeting and potent inhibition of mutant KIT and PDGFRα in GIST

		BLU-285 IC_{50}	Imatinib IC_{50}
KIT Exon 11 deletion	JM domain mutations	0.6 nM	12 nM
KIT Exon 11 V560G		1 nM	87 nM
KIT Exon 11/13	ATP binding site mutations	11 nM	9160 nM
KIT Exon 11/14		28 nM	19650 nM
KIT Exon 17	Activation loop mutations	<2 nM	60–12750 nM
KIT Exon 17 D816V		0.27 nM	8150 nM
PDGFRα Exon 18 D842V		0.24 nM	759 nM



• High kinome selectivity*



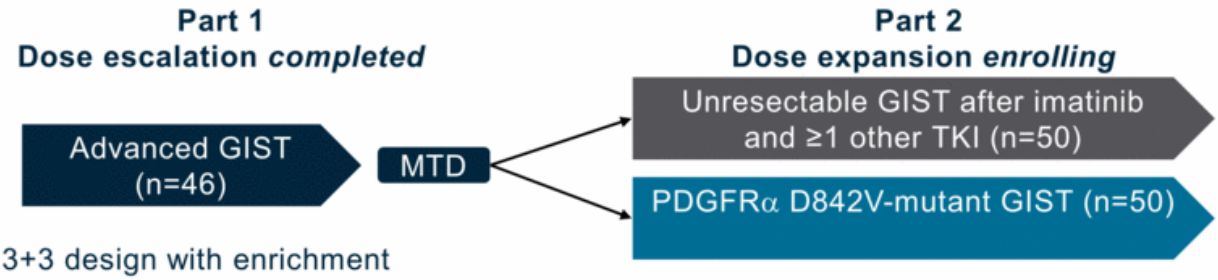
• Binds active conformation

1. Evans EK et al. Sci Transl Med. 2017 Nov 1;9(414)
*Kinome illustration reproduced courtesy of Cell Signaling Technology, Inc. (www.cellsignal.com)

BLU-285 Phase 1 study

Key objectives

- Part 1: MTD, safety, pharmacokinetics, ctDNA analyses, anti-tumor activity
- Part 2: response rate, duration of response, safety



- 3+3 design with enrichment
- MTD determined to be 400 mg PO QD
- RP2D determined to be 300 mg PO QD

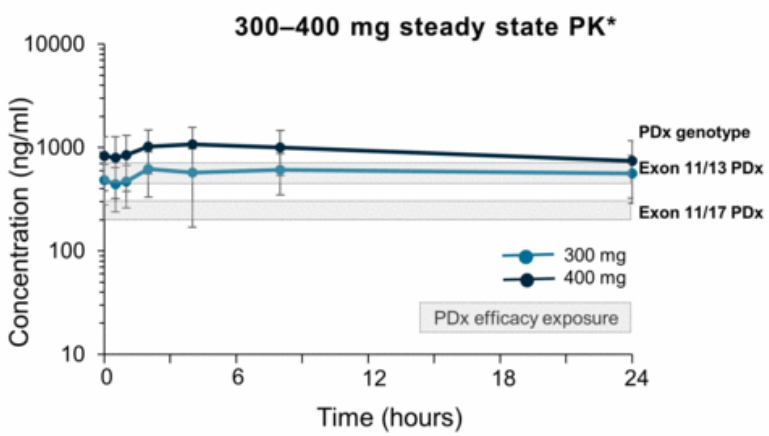
MTD, maximum tolerated dose; RP2D, recommended Phase 2 dose

Demography and baseline patient characteristics

Parameter	All patients, N=116	
Age (years), median (range)	62 (25–85)	
	n (%)	
GIST subtype*		
KIT mutant	76 (66)	
PDGFRα D842 mutant	37 (32)	
PDGFRα Exon 14 (N659K) mutant	2 (2)	
KIT & PDGFRα WT	1 (1)	
Metastatic disease	107 (92)	
Largest target lesion size (cm)		
≤5	27 (23)	
>5–≤10	42 (36)	
>10	46 (40)	
pending	1 (1)	
No. prior kinase inhibitors	PDGFRα	KIT
Median (range)	1 (0–6)	4 (2–11)
≥3	11 (28)	67 (87)
Prior regorafenib	8 (21)	64 (83)

* Data are preliminary and based on a cut off date of 11 Oct 2017

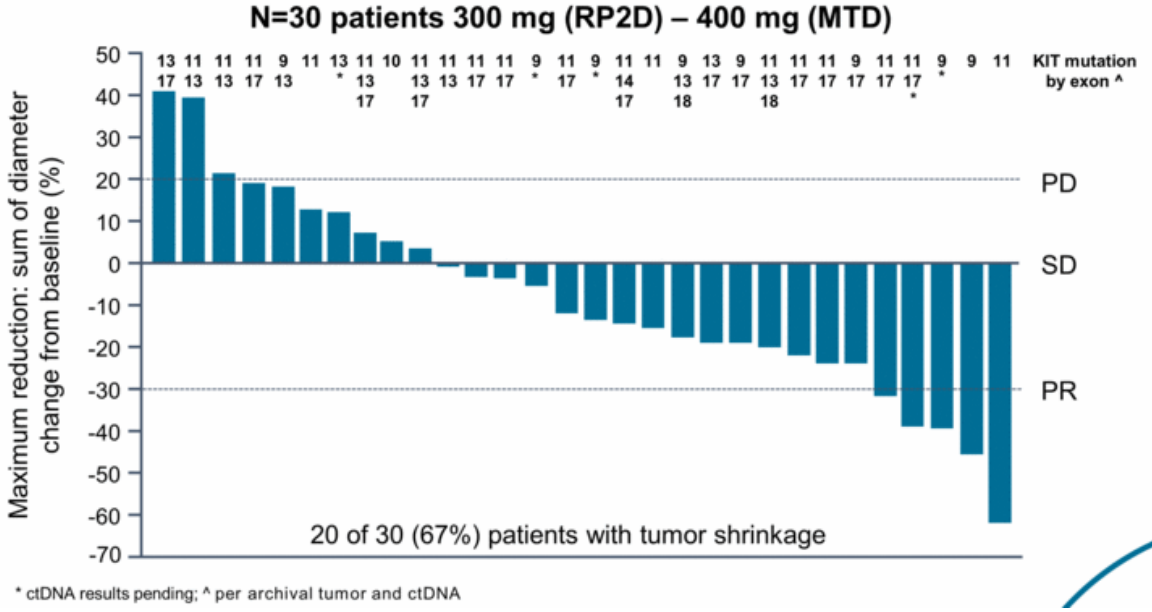
BLU-285 pharmacokinetics support once daily dosing and broad mutational coverage



- Relatively rapid absorption median Tmax 4–6 hours, dose-proportional exposure and long half-life >24 hours
- 300 mg selected as RP2D based on safety, PK, anti-tumor activity

*Includes escalation and expansion data

Tumor reduction across multiple KIT genotypes (central radiology review)



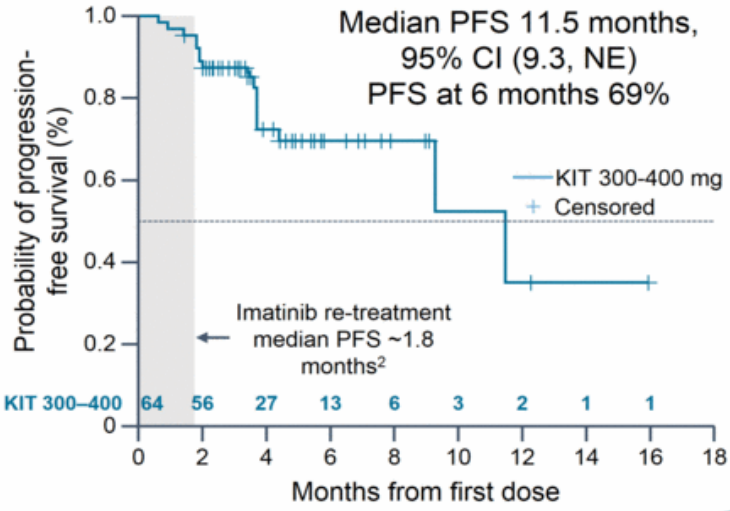
* ctDNA results pending; ^ per archival tumor and ctDNA

Prolonged PFS in heavily pre-treated KIT-mutant GIST (central radiology review)

Best response (N=30)*	Choi Criteria n (%)	RECIST 1.1 n (%)
PR	16 (53)	5 (17)^
SD	7 (23)	18 (60)
DCR (PR+SD)	23 (77)	23 (77)
PD	7 (23)	7 (23)

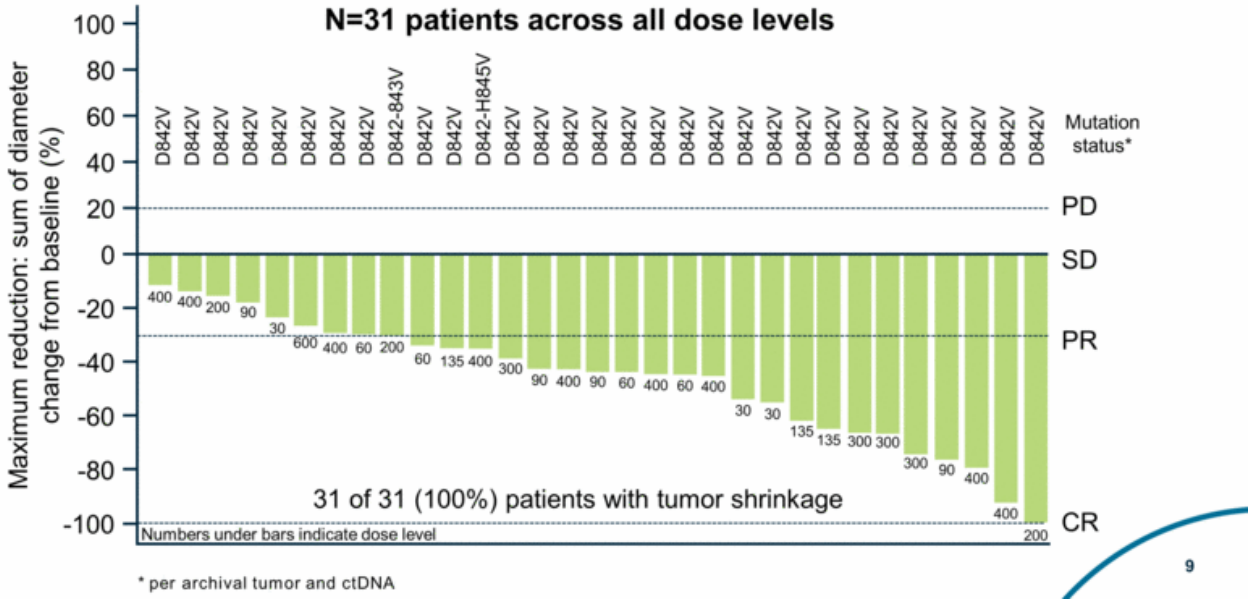
*300 RP2D–400 MTD mg; ^2 pending confirmation

- No approved therapies beyond third-line regorafenib
  - ORR ~0% with imatinib re-treatment in ≥third-line[2]



2. Kang et al. Lancet Oncol. 2013;14(12):1175–82

Remarkable activity in PDGFRα D842-mutant GIST (central radiology review)

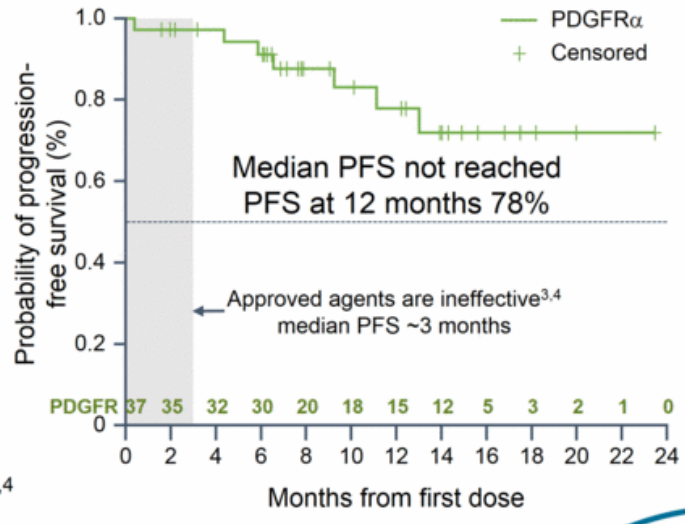


High response rate and prolonged PFS in PDGFRα D842-mutant GIST (central radiology review)

Best response (N=31)*	Choi Criteria n (%)	RECIST 1.1 n (%)
CR	1 (3)	1 (3)^
PR	30 (97)	21 (68)†
CR+PR	31 (100)	22 (71)
SD	0	9 (29)
DCR (PR+SD)	31 (100)	31 (100)
PD	0	0

*All dose levels included
^PR from C3 to C13, CR at C16, CR pending confirmation
†3 pending confirmation

- ORR ~0% with currently approved agents[3,4]



3. Cassier et al. Clin Cancer Res. 2012;18(16):4458–64
4. Yoo et al. Cancer Res Treat. 2016;48(2):546–52

Treatment emergent adverse events ≥20%

Safety population (all patients) N=116		Severity				
Preferred Term, n (%)	Any AE	Grade 1	Grade 2	Grade 3	Grade 4	Grade 5
Nausea	65 (56)	41 (35)	17 (15)	7 (6)	0	0
Fatigue	62 (53)	23 (20)	31 (27)	8 (7)	0	0
Periorbital edema	50 (43)	42 (36)	8 (7)	0	0	0
Vomiting	48 (41)	36 (31)	9 (8)	3 (3)	0	0
Edema peripheral	39 (34)	28 (24)	9 (8)	2 (2)	0	0
Anemia	36 (31)	7 (6)	10 (9)	17 (15)	2 (2)	0
Diarrhea	36 (31)	26 (22)	8 (7)	2 (2)	0	0
Cognitive Effects*	35 (30)	20 (17)	10 (9)	4 (3)	1 (1)	0
Lacrimation increased	35 (30)	29 (25)	6 (5)	0	0	0
Decreased appetite	33 (28)	24 (21)	6 (5)	3 (3)	0	0
Dizziness	27 (23)	21 (18)	6 (5)	0	0	0
Constipation	25 (22)	18 (16)	6 (5)	0	1 (1)	0
Hair color changes	25 (22)	24 (21)	0	0	0	0

* Consists of multiple similar AEs that have been aggregated into a single category. 42% of patients at 400 mg (MTD), 18% of patients at 300 mg (RP2D).

- 39 (34%) patients had grade ≥3 treatment-related AEs: anemia (9%), fatigue (7%), hypophosphatemia (4%), nausea (4%), cognitive effects (3%)
- 67 patients on treatment; 49 discontinued: PD n=40, AEs n=6, withdrew consent n=3

BLU-285 has potent, clinically important activity in GIST

- BLU-285 is well-tolerated at the 300 mg RP2D and provides broad mutational coverage
- Remarkable response rates and prolonged PFS in PDGFRα-driven GIST may support expedited approval path
- Prolonged PFS in heavily pretreated KIT-driven GIST warrants further study, expanding current cohort to 100 patients
- Based on these encouraging data:
  - Second-line expansion cohort has been added and sites are open
  - Phase 3 randomized study comparing BLU-285 to regorafenib in third-line GIST is planned to begin in 1H 2018

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